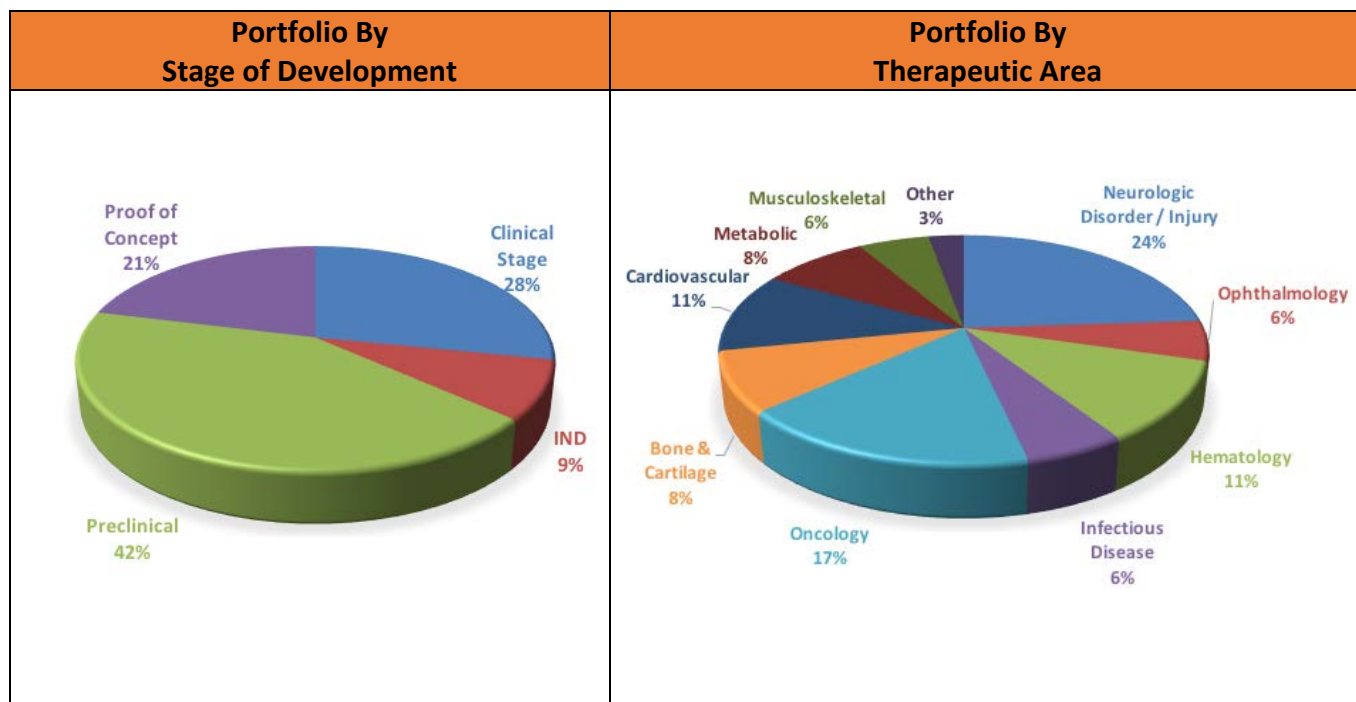


### CIRM's Development Portfolio

There are a total of 71 programs in CIRM's Development Portfolio. These programs are fully funded to complete the phase of development indicated. For ease of reference, we have categorized the portfolio into four broad groups:

- **Clinical Stage Programs.** Include 17 programs currently in the clinic with the goal of completing a Phase 1 through Phase 3 trial, and 3 programs with the dual goal of completing an IND filing and a Phase 1 clinical trial.
- **Investigational New Drug Filing (IND).** Includes 6 programs with the goal of successfully filing an IND with the FDA.
- **Preclinical Stage Programs.** Include 30 programs with the goal of developing a therapeutic candidate ready for IND-enabling preclinical development.
- **Preclinical Proof-of-Concept.** Includes 15 programs with the goal of achieving initial preclinical proof of concept for a therapeutic development candidate.



### Additional Information

Additional information regarding individual programs can be found on the CIRM website: [www.cirm.ca.gov](http://www.cirm.ca.gov). Simply type the name of the company or principal investigator in the search bar located at the top right of the web page.

In addition, we encourage you to directly contact the principal investigators, or contact:

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## NEUROLOGIC DISORDERS & INJURY

Neurologic Injuries						
Company/Principal Investigator	Disease / Injury	Phase of Development	Therapeutic Modality	Therapeutic Cell Type	Approach	Total CIRM Funding
Asterias Biotherapeutics	Spinal Cord Injury	Phase 1/2a	Cell Therapy	Oligodendrocyte progenitor	Allogeneic	\$14.3M
Lipton, Scintillon Institute	Stroke	Preclinical Proof-of-Concept	Genetically Modified Cell Therapy	Neural progenitor	Allogeneic	\$2.1M

Neurodegenerative Disorders						
Company/Principal Investigator	Disease / Injury	Phase of Development	Therapeutic Modality	Therapeutic Cell Type	Approach	Total CIRM Funding
Svendson, Cedars-Sinai	ALS	IND, Phase 1	Genetically Modified Cell Therapy	Neural progenitor	Allogeneic	\$17.0M
Yeo, UCSD	ALS	Preclinical Proof-of-Concept	Small Molecule	N/A	N/A	\$1.5M
Finkbeiner, Gladstone	ALS	Preclinical Proof-of-Concept	Small Molecule	N/A	N/A	\$2.3M
Goldstein, UCSD	ALS	Preclinical	Cell Therapy	hESC	Allogeneic	\$6.3M
Schubert, Salk	Alzheimer's Disease	Preclinical	Small Molecule	N/A	N/A	\$1.7M
Goldstein, UCSD	Alzheimer's Disease	Preclinical Proof-of-Concept	Small Molecule	N/A	N/A	\$1.7M
Walsh, UCI	Multiple Sclerosis	Preclinical	Cell Therapy	N/A	Allogeneic	\$4.5M
Schultz, Scripps	Multiple Sclerosis	Preclinical	Small Molecule	N/A	N/A	\$4.3M
Thompson, UC Irvine	Huntington's Disease	Preclinical	Cell Therapy	Neural stem cells	Allogeneic	\$8.9M
Grikscheit, CHOLA	Enteric neuropathies	Preclinical	Cell Therapy	iPSC	Allogeneic	\$7.1M

Pediatric Neurologic Disorders						
Company/Principal Investigator	Disease / Injury	Phase of Development	Therapeutic Modality	Therapeutic Cell Type	Approach	Total CIRM Funding
Farmer, UC Davis	Spina bifida	Preclinical	Cell Therapy	Placental MSCs	Allogeneic	\$2.2M
Schwartz, CHOC	Lysosomal Storage Disease	Preclinical	Cell Therapy	HSC & iPSC	Allogeneic	\$5.5M
Muotri, UCSD	Autism Spectrum Disorder	Preclinical Proof-of-Concept	Small Molecule	N/A	N/A	\$1.8M
Anderson, UCD	Tay-Sachs and Sandhoff disease	Preclinical	Cell Therapy	HSC	Autologous	\$.88M
Shi, COH	Canavan disease	Preclinical	Cell Therapy, Combination	iPSC	Autologous	\$7.3M

## OPHTHALMOLOGY

Ophthalmology						
Company/Principal Investigator	Disease / Injury	Phase of Development	Therapeutic Modality	Therapeutic Cell Type	Approach	Total CIRM Funding
Humayun, USC	Age-related macular degeneration (dry form)	Phase 1	Cell Therapy, Combination	Retinal pigment epithelial	Allogeneic	\$18.9M
Wang, Cedars-Sinai	Retinitis Pigmentosa	IND	Cell Therapy	Neuronal progenitor	Allogeneic	\$4.6M
Klassen, UC Irvine	Retinitis Pigmentosa	Phase 1/2a	Cell Therapy	Retinal Progenitor cells	Allogeneic	\$17.3M
Seiler, UC Irvine	Retinitis Pigmentosa, AMD (dry form)	Preclinical	Cell Therapy	Retinal pigment epithelial	Allogeneic	\$4.3M

## HEMATOLOGY

Hematology						
Company/Principal Investigator	Disease / Injury	Phase of Development	Therapeutic Modality	Therapeutic Cell Type	Approach	Total CIRM Funding
Kohn UCLA	X-linked Chronic Granulomatous Disease.	Phase 1/2	Genetically Modified Cell Therapy	Hematopoietic stem cells	Autologous	\$7.4M
Kohn, UCLA	Sickle Cell Disease	Phase 1	Genetically Modified Cell Therapy	Hematopoietic stem cells	Autologous	\$13.9M
Shizuru, Stanford	X-linked severe combined immunodeficiency	IND, Phase 1/2a	Biologic	N/A	N/A	\$20.0M
Porteus, Stanford	Severe combined immunodeficiency	Preclinical	Genetically Modified Cell Therapy	Hematopoietic stem cells	Autologous	\$1.0M
Cowan, UCSF	Severe combined immunodeficiency	Preclinical	Genetically Modified Cell Therapy	Hematopoietic stem cells	Autologous	\$3.9M
Puck, UCSF	Severe combined immunodeficiency	IND	Genetically Modified Cell Therapy	Hematopoietic stem cells	Autologous	\$4.2M
Kohn, UCLA	Sickle Cell Disease	Preclinical Proof-of-Concept	Genetically Modified Cell Therapy	Hematopoietic stem cells	Autologous	\$1.8M
Verma, Salk	Hemophilia B	Preclinical Proof-of-Concept	Genetically Modified Cell Therapy	iPSC derived hepatocytes	Autologous	\$2.3M

## INFECTIOUS DISEASE

Infectious Disease						
Company/Principal Investigator	Disease / Injury	Phase of Development	Therapeutic Modality	Therapeutic Cell Type	Approach	Total CIRM Funding
Calimmune	HIV/AIDS	Phase 1/2a	Genetically Modified Cell Therapy	Hematopoietic stem cells	Autologous	\$8.3M
Abedi, UC Davis	HIV/AIDS	Phase 1	Genetically Modified Cell Therapy	Hematopoietic stem cells	Autologous	\$7.4M
Zaia, City of Hope	HIV/AIDS	Phase 1	Genetically Modified Cell Therapy	Hematopoietic stem cells	Autologous	\$5.6M
Zack, UCLA	HIV/AIDS	Preclinical	Genetically Modified Cell Therapy	Hematopoietic stem and/or Tscm cells	Autologous	\$5.3M

## ONCOLOGY

Oncology: Hematologic Malignancies						
Company/Principal Investigator	Disease / Injury	Phase of Development	Therapeutic Modality	Therapeutic Cell Type	Approach	Total CIRM Funding
Kipps, UCSD	Chronic lymphocytic leukemia	Phase 1	Biologic	N/A	N/A	\$4.2M
Weissman, Stanford	AML and Solid Tumor	Phase 1	Biologic	N/A	N/A	\$12.7M
Müschen, UCSF	AML, ALL	Preclinical	Small Molecule	N/A	N/A	\$3.6M
Angiocrine Bioscience	Hematologic malignancies including leukemia and lymphoma	IND, Phase 1	Cell Therapy, Combination	HSPC	Allogeneic	\$3.7M

Oncology: Solid Tumors						
Company/Principal Investigator	Disease / Injury	Phase of Development	Therapeutic Modality	Therapeutic Cell Type	Approach	Total CIRM Funding
Immunocellular Therapeutics	Glioblastoma	Phase 3	Biologic	N/A	Autologous	\$20.0
Ribas, UCLA	Melanoma	IND, Phase 1	Genetically Modified Cell Therapy	Hematopoietic stem cells	Autologous	\$20.0M
Slamon, UCLA	Solid Tumor	Phase 1	Small Molecule	N/A	N/A	\$6.9M
Kasahara, UCLA	Glioblastoma	Preclinical	Genetically Modified Cell Therapy + Small Molecule	Mesenchymal stem cells	Allogeneic	\$3.4M
Forman, City of Hope	Glioblastoma	Preclinical	Genetically Modified Cell Therapy	Memory T Cells	Autologous	\$5.2M
Wong, Stanford	Glioblastoma	Preclinical	Biologic	Peptide	N/A	\$2.9M
Reiter, UCLA	Prostate cancer	Preclinical	Biologic	N/A	N/A	\$4.1M
Yang, UCLA	Solid Tumor	Preclinical	Genetically Modified Cell Therapy	T-cell	Autologous	\$7.6M



## **BONE & CARTILAGE DISORDERS**

<b>Bone &amp; Cartilage</b>						
<b>Company/Principal Investigator</b>	<b>Disease / Injury</b>	<b>Phase of Development</b>	<b>Therapeutic Modality</b>	<b>Therapeutic Cell Type</b>	<b>Approach</b>	<b>Total CIRM Funding</b>
Lane, UC Davis	Osteoporosis	IND, Phase 1/2a	Small Molecule	N/A	N/A	\$20.0M
D'Lima, Scripps	Osteochondral defects	Preclinical	Cell Therapy, Combination	Chondro-progenitor cells	Allogeneic	\$7.7M
Gazit, Cedars-Sinai	Bone fractures	Preclinical	Gene Therapy	N/A	N/A	\$5.2M
Schultz, Caliber	Osteoarthritis, cartilage injuries	IND	Small Molecule	N/A	N/A	\$1.7M
Schultz, California Inst. For Biomedical Research	Osteoarthritis	IND	Small Molecule	N/A	N/A	\$2.6M
Athanasiou, UC Davis	Articular cartilage defects	Preclinical Proof-of-Concept	Cell Therapy	Dermis isolated adult stem cells	Autologous	\$1.7M

## CARDIOVASCULAR

Cardiovascular						
Company/Principal Investigator	Disease / Injury	Phase of Development	Therapeutic Modality	Therapeutic Cell Type	Approach	Total CIRM Funding
Capricor	Heart dysfunction after MI/Chronic heart failure	Phase 2	Cell Therapy	Cardiac derived stem cells	Allogeneic	\$19.8M
Wu, Stanford	End stage heart failure	IND	Cell Therapy	Cardiomyocyte	Allogeneic	\$20.0M
Wu, Stanford	Cardiovascular Disease	Preclinical	Cell Therapy, Combination	Cardiomyocyte	Allogeneic	\$4.8M
Srivastava, Gladstone	Cardiovascular Disease	Preclinical	Direct Reprogramming (in vivo)	N/A	N/A	\$6.3M
Cashman, Human Biomolecular Research Institute	Ventricular arrhythmias	Preclinical	Small Molecule	N/A	N/A	\$6.4M
Xu, UCSD	Cardiovascular Disease	Preclinical Proof-of-Concept	Genetically Modified Cell Therapy	Cardiomyocyte	Allogeneic	\$1.9M
Izpisua Belmonte, Salk	Cardiovascular , Vascular Disease	Preclinical Proof-of-Concept	Cell Therapy	Vascular progenitor cells	Autologous	\$2.3M
Adler, UCSD	Danon disease	Preclinical Proof-of-Concept	Small Molecule	N/A	N/A	\$1.7M

## **METABOLIC DISORDERS**

<b>Metabolic Disorders</b>						
<b>Company/Principal Investigator</b>	<b>Disease / Injury</b>	<b>Phase of Development</b>	<b>Therapeutic Modality</b>	<b>Therapeutic Cell Type</b>	<b>Approach</b>	<b>Total CIRM Funding</b>
ViaCyte	Type 1 Diabetes	Phase ⅔a	Encapsulated Cell Therapy	Pancreatic progenitor	Allogeneic	\$26.7M
Isseroff, UC Davis	Diabetic foot ulcers	Preclinical	Cell Therapy, Combination	Mesenchymal stem cell	Allogeneic	\$9.7M
Miki, USC	Liver Disease, Congenital	Preclinical Proof-of-Concept	Cell Therapy	Amniotic epithelial cells	Allogeneic	\$1.8M
Lipshutz, UCLA	Arginase Deficiency	Preclinical Proof-of-Concept	Genetically Modified Cell Therapy	iPSC- derived Hepatocytes	Autologous	\$1.8M
Willenbring, UCSF	Liver Disease, Chronic	Preclinical Proof-of-Concept	Genetically Modified Cell Therapy	iHep	Autologous	\$1.5M
Semma Therapeutics	Type 1 Diabetes	Preclinical	Cell Therapy	iPSC	Autologous	\$5M

## MUSCULOSKELETAL

Musculoskeletal						
Company/Principal Investigator	Disease / Injury	Phase of Development	Therapeutic Modality	Therapeutic Cell Type	Approach	Total CIRM Funding
Nelson, UCLA	Duchenne muscular dystrophy	Preclinical Proof-of-Concept	Combination	N/A	N/A	\$1.8M
Capricor	Duchenne muscular dystrophy cardiomyopathy	Phase 2	Cell Therapy	Cardiosphere-Derived Cells (CDCs)	Allogeneic	\$3.3M
Calos, Stanford	Limb Girdle Muscular Dystrophy Type 2B	Preclinical Proof-of-Concept	Genetically Modified Cell Therapy	Skeletal muscle stem cells	Autologous	\$1.9M
Blau, Stanford	Age-related Muscle Atrophy	Preclinical Proof-of-Concept	Cell Therapy	Muscle stem cells	Autologous	\$3.3M

## OTHER

Other						
Company/Principal Investigator	Disease / Injury	Phase of Development	Therapeutic Modality	Therapeutic Cell Type	Approach	Total CIRM Funding
Belafsky, UC Davis	Airway stenosis	IND	Cell Therapy, Combination	Bone Marrow Stromal + Airway epithelial	Autologous	\$4.4M
Chen, Stanford	Urinary Incontinence	Preclinical	Cell Therapy, Combination	Smooth muscle progenitors	Autologous	\$5.3M